CASE

Treatment Access for HIV/AIDS Patients

Challenge:

People with HIV/AIDS face a life-threatening disease that, when it first reached epidemic proportions, had few promising treatment options. In the early 1990s, experimental therapies, such as protease inhibitors, were not widely accessible to those in greatest need because of the premium placed by regulatory review procedures on determining whether a new treatment is both safe and effective. As patients increasingly asked to receive emerging treatments before the clinical trials and regulatory approvals were completed, U.S. Food and Drug Administration (FDA) officials and others began to recognize a need to rethink how society provides both the greatest access and protection to those in need.

Result:

The Keystone Center convened a national dialogue on expanded access to therapeutic drugs for HIV/AIDS. FDA was reconsidering its regulations for protease inhibitors at this time, considering the implications for other life-threatening diseases, and issued new regulations informed by their participation in the dialogue. Patient access to experimental treatment options was increased both during clinical trials and by making drugs available in the marketplace sooner by expediting the approval process. The shared vision was that patients would get increased access to therapies and the revenues from earlier release in the marketplace would spark new research on treatment options.

Participants

ActUp New York
AIDS Action Council
Association of American Universities
Black Leadership Commission on AIDS
Blicker Futterman and Stein
Body Positive Resource Center, Inc.
Burroughs Wellcome Company
Community Research Initiative of New England
Gay Men's Health Crisis
Genentech Incorporated
Genetics Institute
Glaxo Incorporated
GTE Service Corporation
Health Care Financing Administration
Health Insurance Association of America
Mario Solis-Marich and Associates
Medical Services Administration
Medicus Public Relations
Merck & Company Incorporated
Merck Research Laboratories
Merck, Sharp & Dohme
Michael Carbine
Michaels and Wishner, P.C.
National Cancer Institute
National Commission on AIDS
National Institutes of Health/Division of AIDS
Nationwide Insurance Company
Office of Congressman Sander M. Levin
People of Color Against AIDS Network
Pharmaceuticals Manufacturers Association
Project Inform
San Francisco General Hospital
Stanford University/Division of Infectious Diseases
Syntex Research
The Orphan Project/Fund for the City of New York
U.S. Food and Drug Administration
U.S. House Energy and Commerce Committee
U.S. Office of Management and Budget
U.S. Office of Technology Assessment
University of Michigan School of Public Health
University of San Diego Medical Center

MEDIATOR
Abby Dilley, The Keystone Center (now at RESOLVE)

Issues

The rise of the HIV/AIDS epidemic raised new questions about policies for ensuring that medical treatment options are safe and effective, while getting new drugs to the patients that need them. People with HIV/AIDS face a life-threatening disease that, in the early days of the epidemic, had few promising treatment options. As patients increasingly asked to receive emerging treatments before the clinical trials and regulatory approvals were completed, U.S. Food and Drug Administration (FDA) officials and others began to recognize a need to rethink how society provides both the greatest access and protection to those in need.

In the early 1990s, experimental therapies, such as protease inhibitors, were not widely accessible. Some patients who were not responding to the drugs commercially available at the time were willing to try emerging treatments before full agency approval because earlier access to these drugs offered them their best hope. However, traditional regulatory restrictions to early access to treatments and clinical research processes are not designed for this type of access. It can take years and millions of dollars to get a medicine to market, and clinical trials have typically been designed with narrow entry criteria that limit broad patient access to new therapies. Other issues such as financing, general medical care access and patient access to full information also present barriers to giving patients who lack good alternatives other opportunities to improve their quality of life.

Process

In response to the growing desire to overcome some of these barriers, The Keystone Center convened a national dialogue on expanded access to therapeutic drugs for HIV/AIDS. The goal of the project was to clarify issues and explore new policy options for expedited development and broadened pre-approval access to promising therapeutic drugs for AIDS. In exploring mechanisms for expanded access, participating groups began with a shared desire to give patients opportunities for greater quality of life while continuing to pursue clinical research on promising therapies. The dialogue participants developed a range of recommendations, including improvements in education and information efforts, adjustments in clinical research design to support expanded access, and ways to address the finance and resource issues.

Results

FDA was in the process of reconsidering its regulations for protease inhibitors at this time. The dialogue was not established formally to provide advice to FDA, but the ideas that emerged from the dialogue informed all stakeholders' contributions to the new regulations. "Parallel track," announced in 1992 as a policy statement of the U.S. Public Health Service Agency, permits the distribution of drugs for HIV during the development phase when there is a lack of satisfactory alternative therapies. Parallel track drugs are distributed under a protocol which is run "parallel" to the clinical trial being conducted to collect data necessary for approval.

Patient access to experimental treatments options was increased both during clinical trials and by making drugs available in the marketplace sooner by expediting the approval process. The shared vision was that patients would get increased access to therapies and the revenues from earlier release in the marketplace would spark new research on treatment options.

The dialogue group recognized that the issues and approaches discussed seem broadly applicable to other life-threatening disease settings where therapeutic options are limited, such as Alzheimer's disease and cancer. The group also felt that any progress concerning policy on expanded access would contribute to the future structure of an improved health care system.

Stakeholders, including those engaged in this dialogue, continued to monitor health outcomes for HIV patients on treatment regimens, including therapeutics made available through expanded access, to ensure optimization of the medical management of HIV. After several years of experience, significant information was being accumulated on the potential for unintended consequences of these therapeutics and treatment options.

The Forum for Collaborative HIV Research (Forum) was founded in 1996, as a result of a subsequent dialogue that included many stakeholders involved in this project. The Forum is a public-private partnership to serve as a catalyst for research to address key questions and to enhance treatment protocols. Agreement to launch the Forum was the result of a four-month-long, intensive dialogue involving many of those who had been critical to the earlier process. Abby Dilley has continued to facilitate Forum meetings as well as other policy dialogues on HIV/AIDS.

Scientific/Technical Obstacles and Actions

OBSTACLE	ACTION
Information had become politicized	Participants talked explicitly about trust, uncertainty, and values
Key stakeholders defined the problem differently and valued different aspects of the issues, in part because the conceptual framework for the problem was shifting	Participants named these differences and used them as a context for substantive conversations. Detailed discussions about the different phases of experimental therapeutics, allowed them to think together about what opportunities existed for increased access, what the impacts would be, and how to mitigate them

RESOLVE, Inc.
1255 23rd Street, NW, Suite 275, Washington, DC 20037
Ph: 202-944-2300 Fax: 202-338-1264
www.resolv.org